

205THO" 62226650

Non-Transduced hMSCs in Immuno-competent mouse muscle

Day 3

DiI + hMSCs

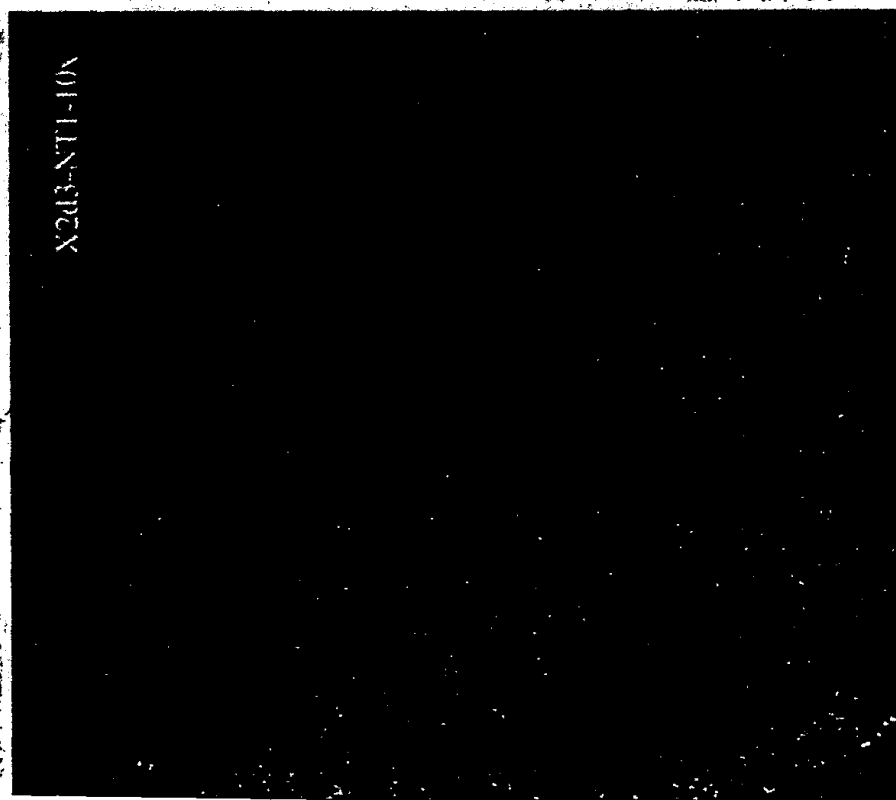


FIG. 1α

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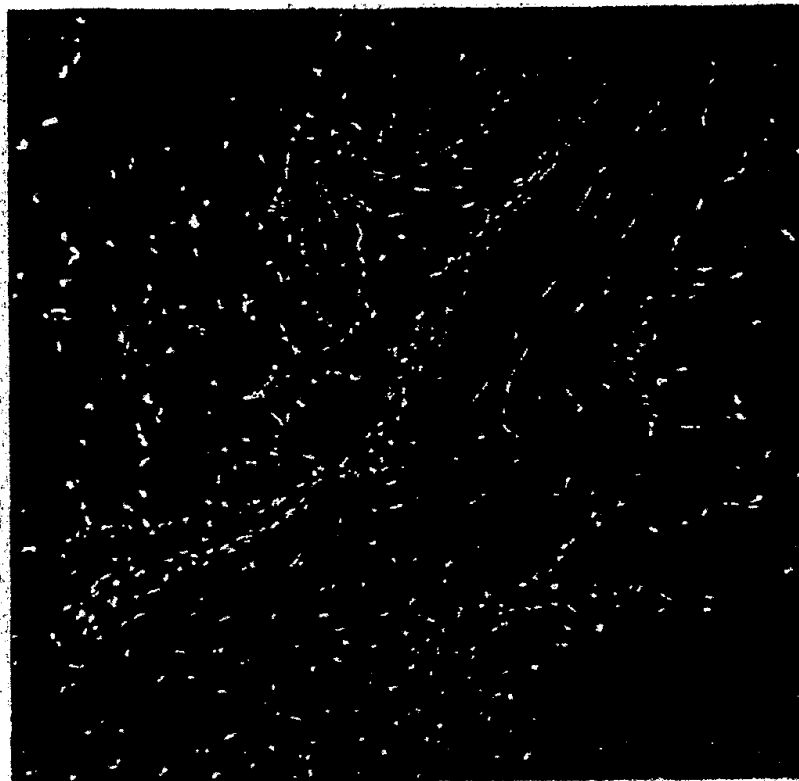
FIG. 1b

Human MSCSs in Mouse Muscle: Day 7

(H&E)



DiI labelled cells +
Sybr-green staining
of all nuclei



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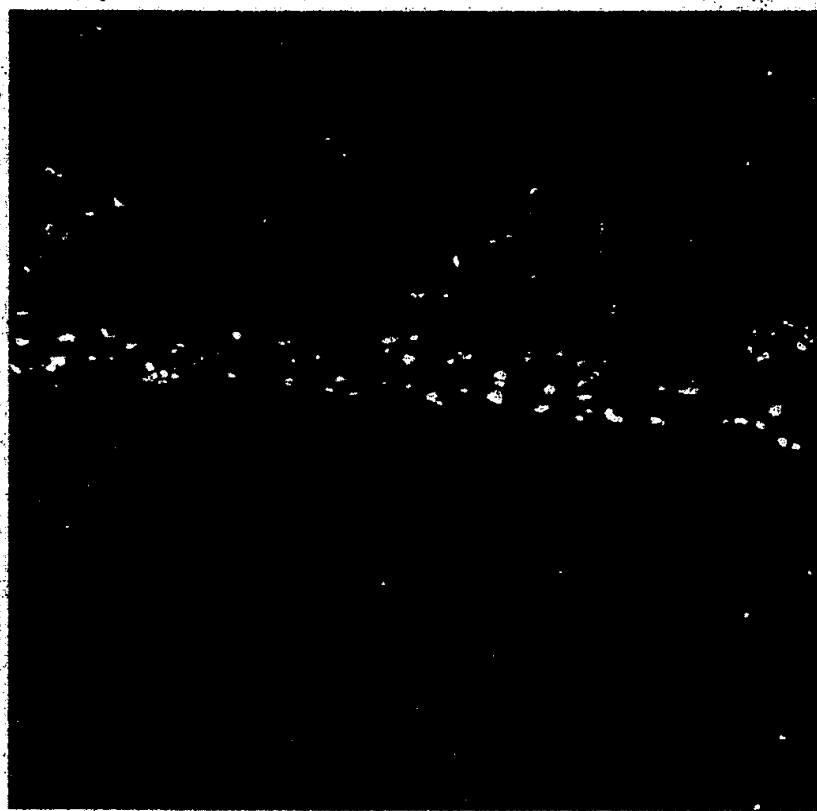
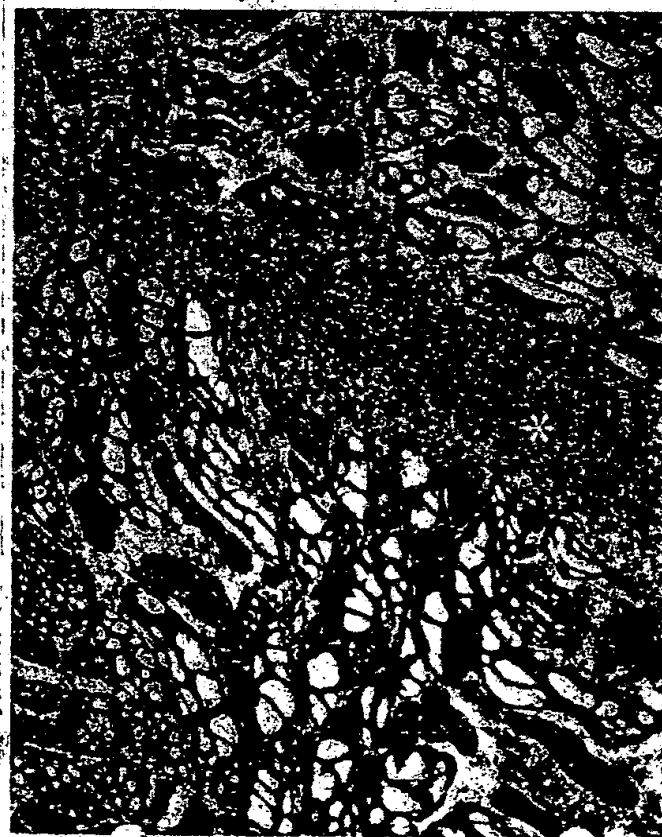
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+

FIG. 1c

Human MSCs in Mouse Muscle: Day-7

(H&E, 20X)



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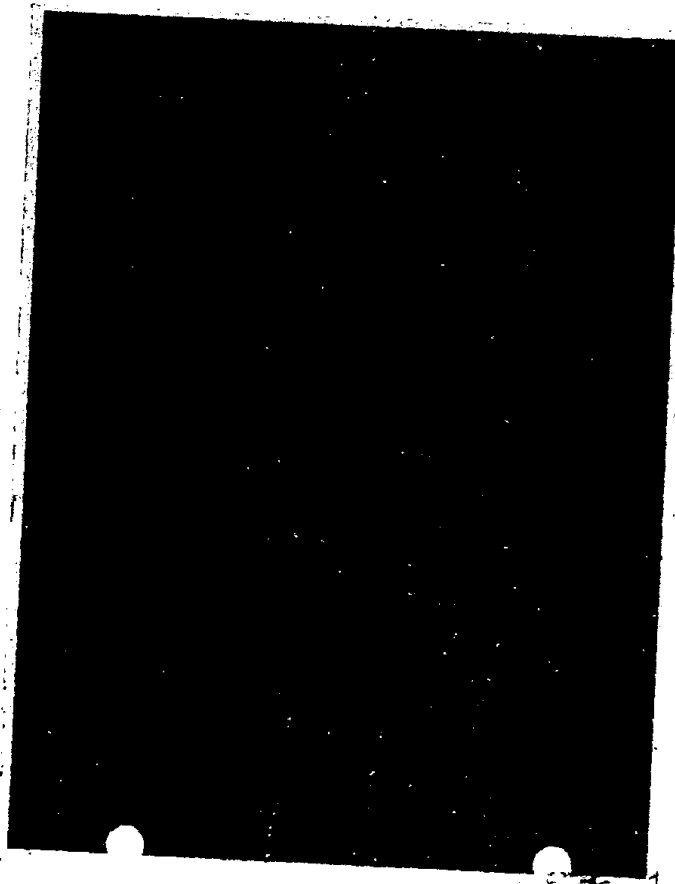
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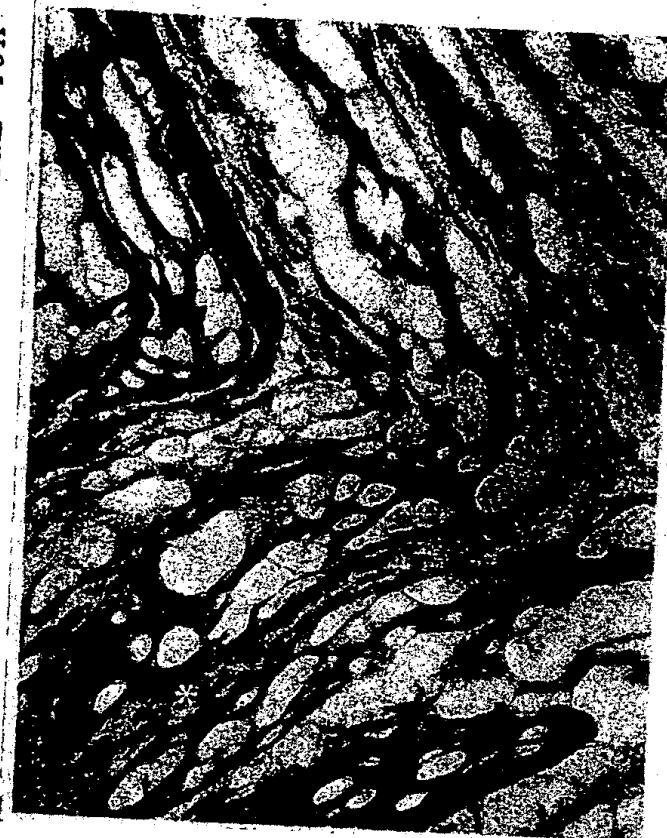
FIG. 1d

Human MSCs in Mouse Muscle: Day 14

Fluorescent Field, 10X



H&E 40X



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FIG. 1e

DiI-Positive d-Gala Transduced hMSCs in
Immuno-competent mouse muscle

d29



d43



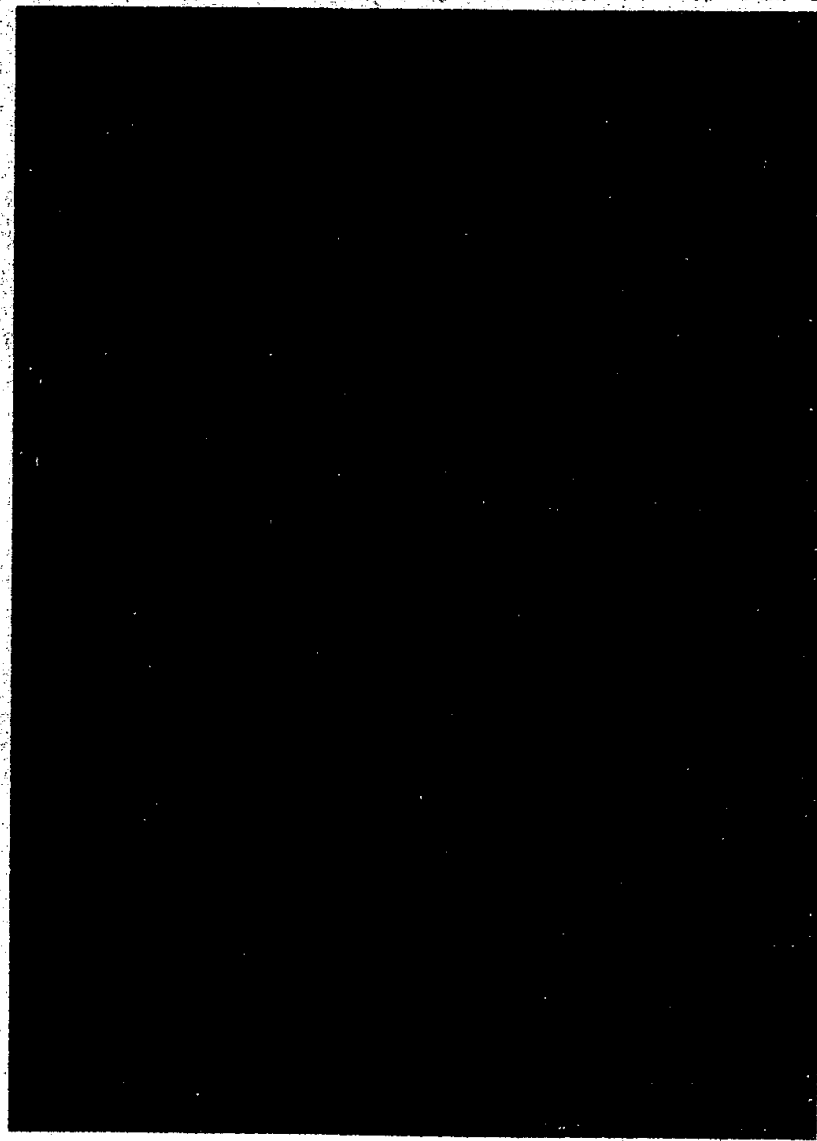
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FIG. 1f

Human α GalA-Transduced MSCs in Mouse Muscle: Day-57

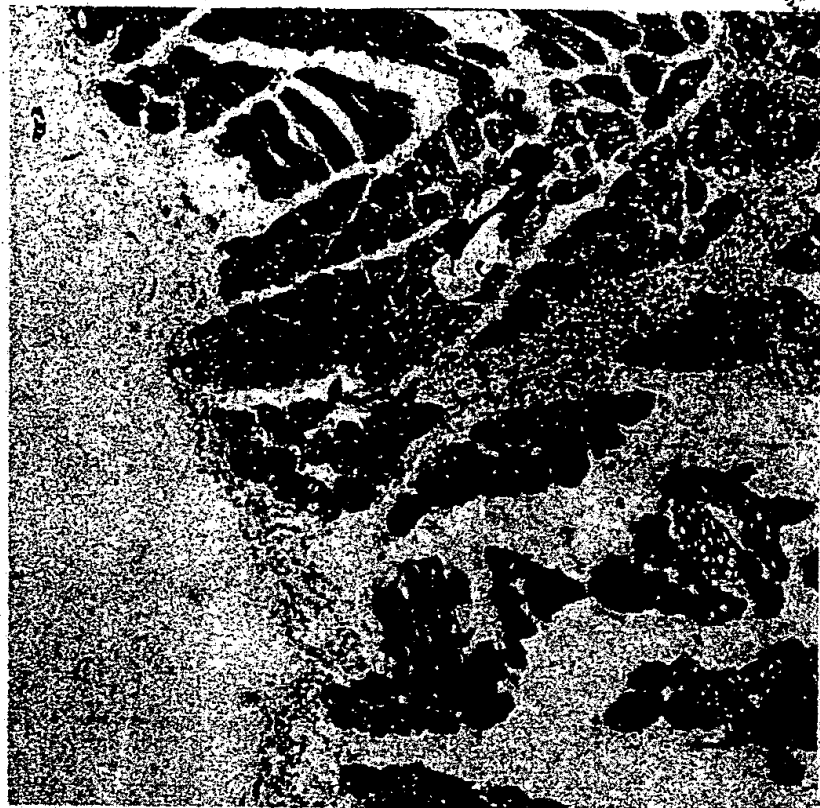
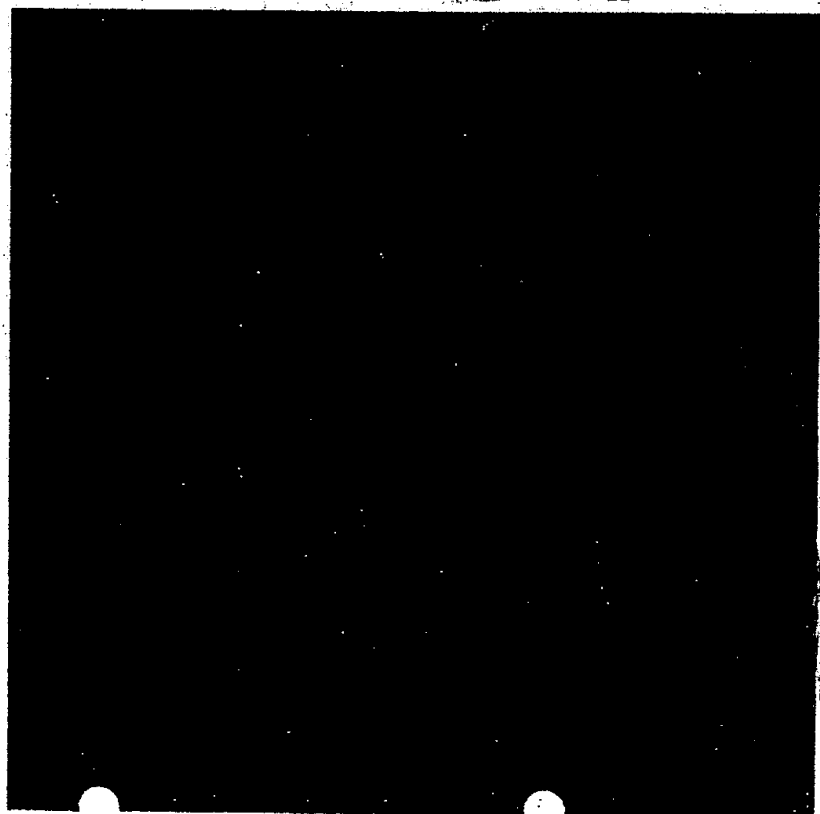
10X



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FIG. 2

D3: IM injection of DiI-labeled PBMCs



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FIG. 3

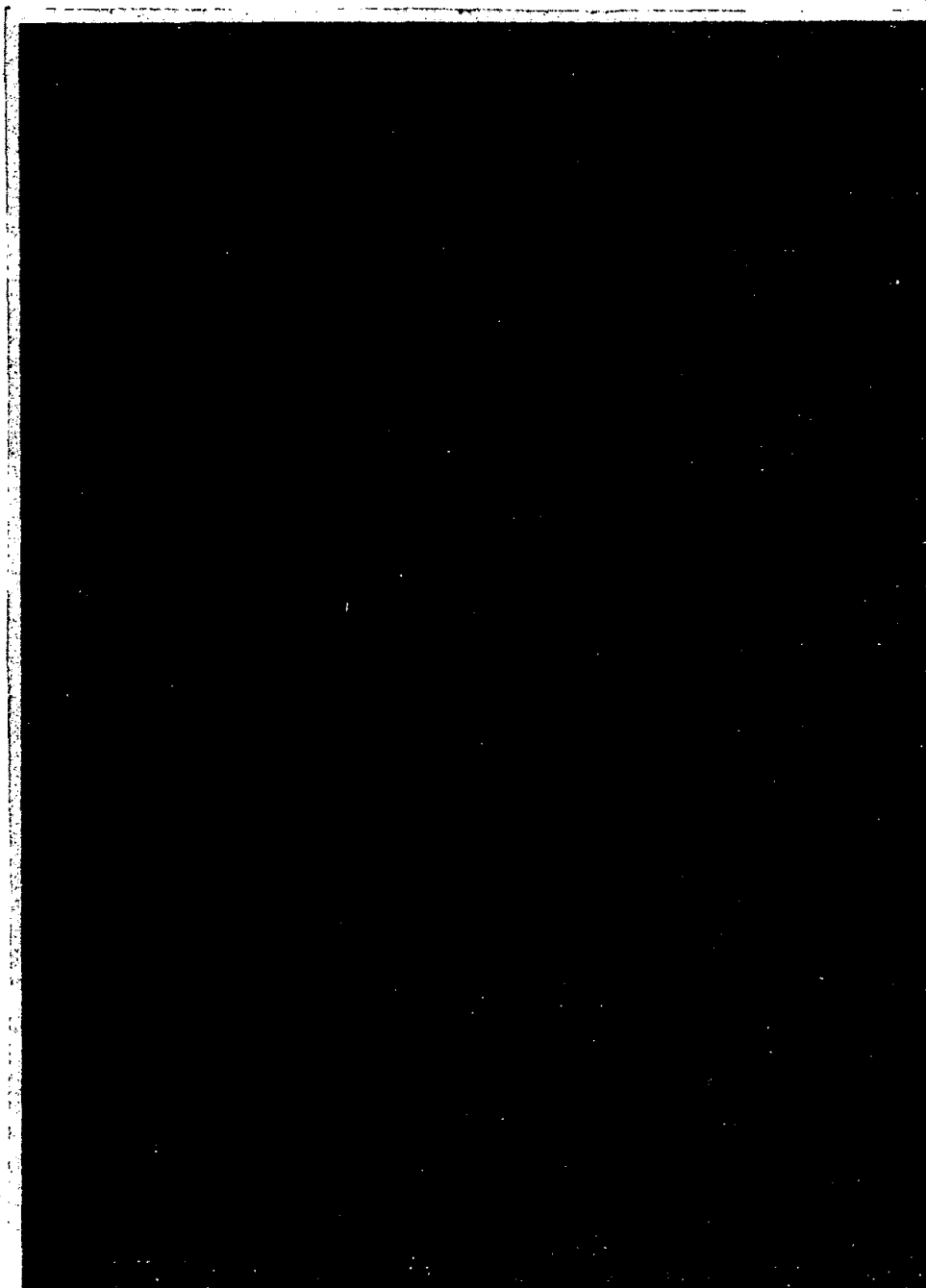
D3 IM injection of saline



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FIG. 4

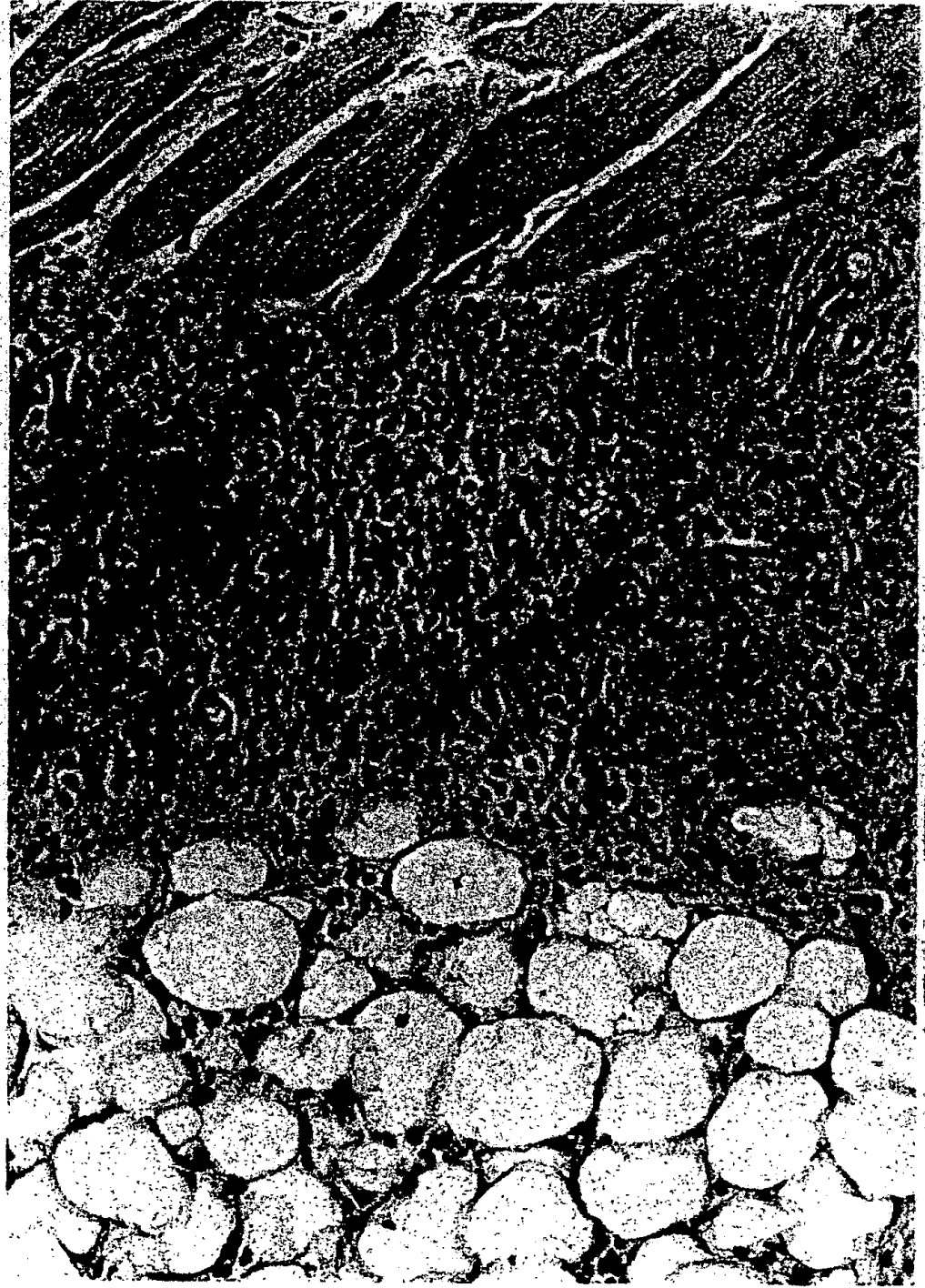
Red and Blue Florescence Channels merged



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FIG. 5

α GalA Immunostain of Muscle from Mouse
injected IM with α GalA-hMSCs: 14 days

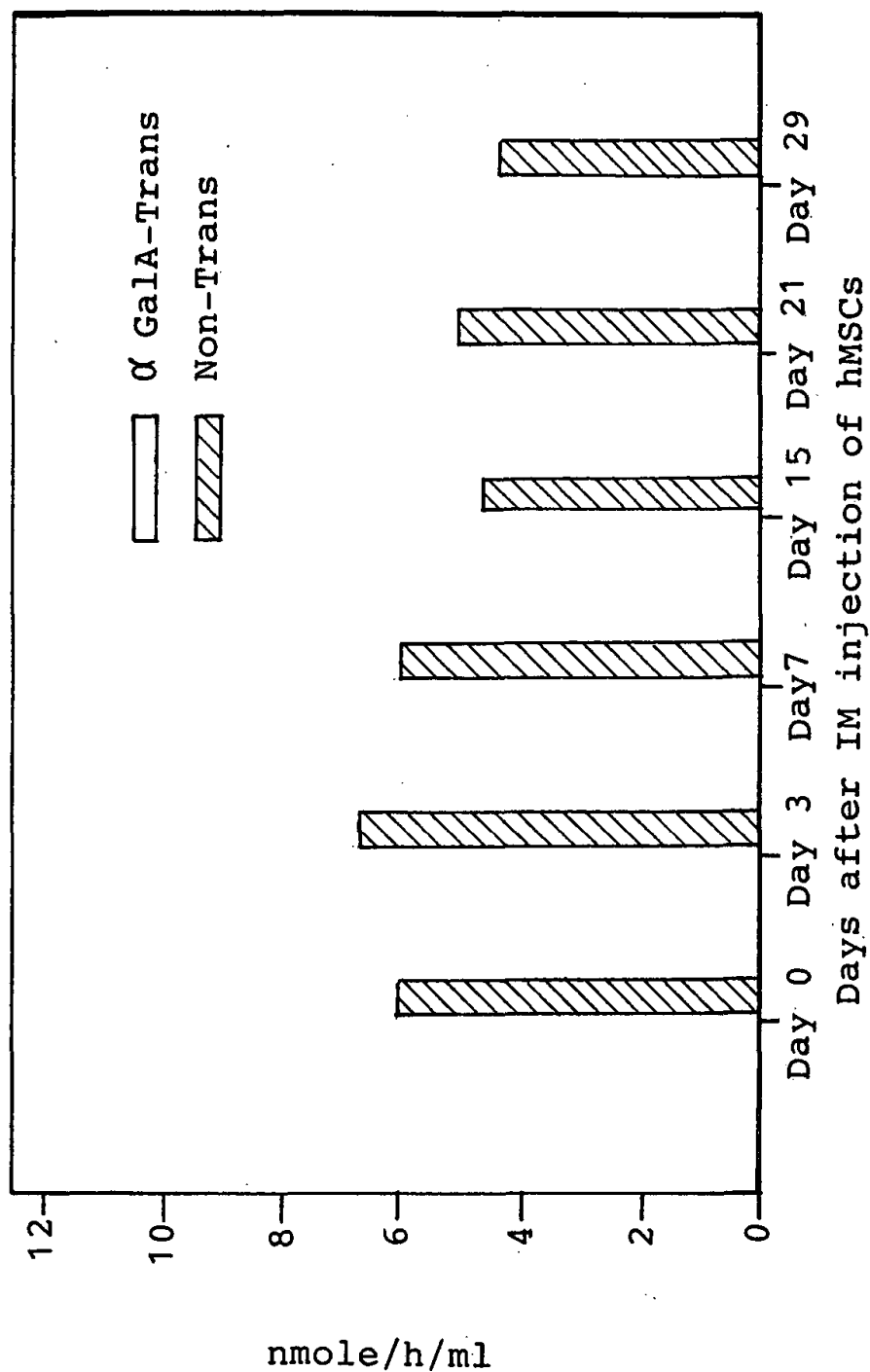


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FIG. 6

α -Gala activity in Plasma of Mice
IM-injected with α -Gala-Transduced h MSCs



#: NO MSCs, *n=2,

Fabry Mice plasma: 0.5 nmole/h/ml, PNAS 97:7515

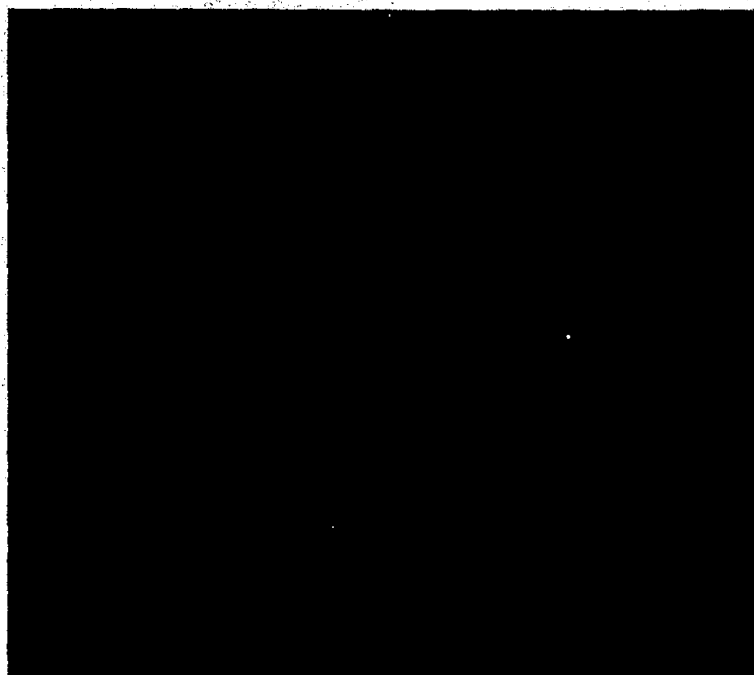
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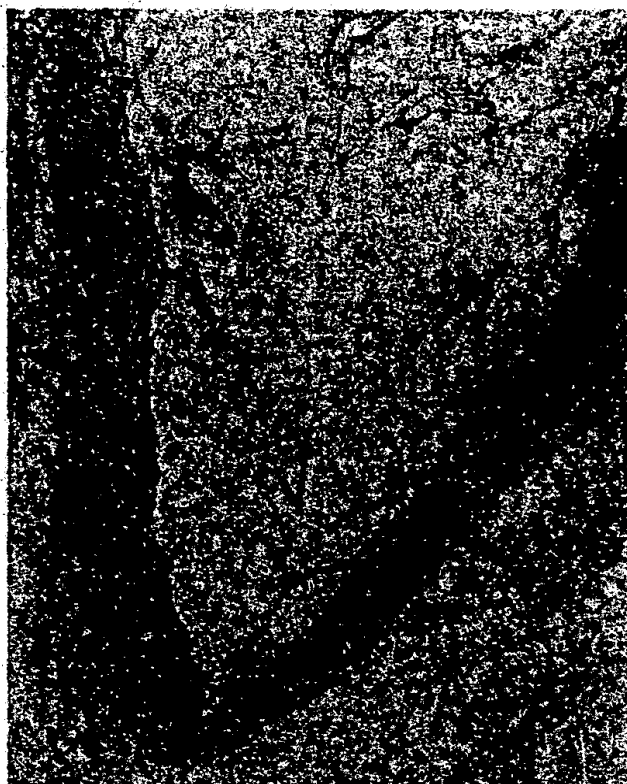
FIG. 7a

Human-specific Alu esquence staining of hMSCs
injected into Immuno-competent mouse muscle

Dil-positive cells



Alu-sequence positive cells



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FIG. 7b

Human MSCs in Mouse Muscle: 43 days

H & E



Dil Positive Cells



Alu-Positive cells



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Day 57 DiI/Alu

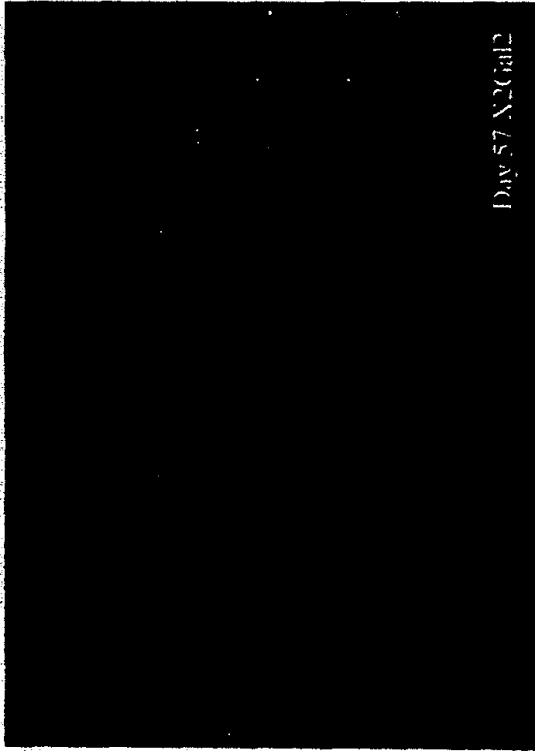


FIG. 7c

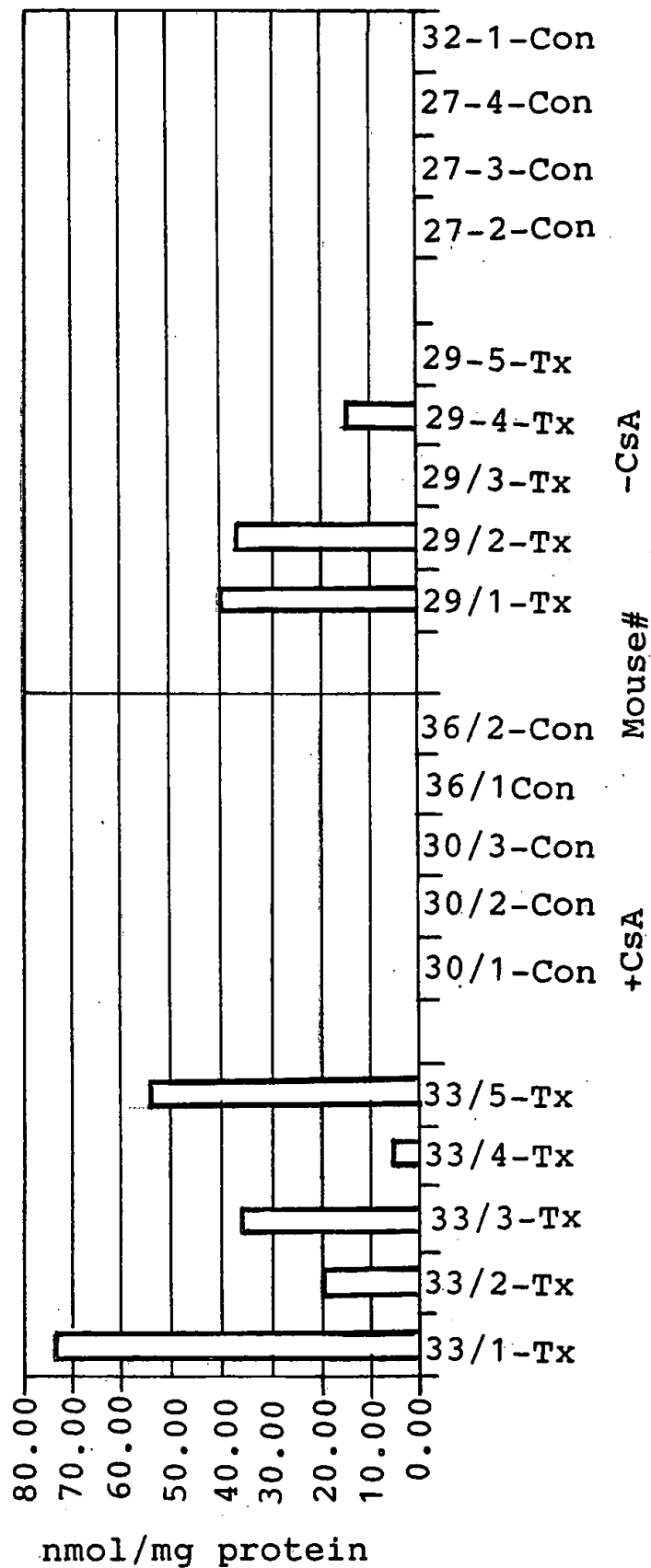
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FIG. 8a

α Gala Activity in Muscles of Fabry-KO mice:
14 days after IM injection of α Gala-Transduced hMSCs

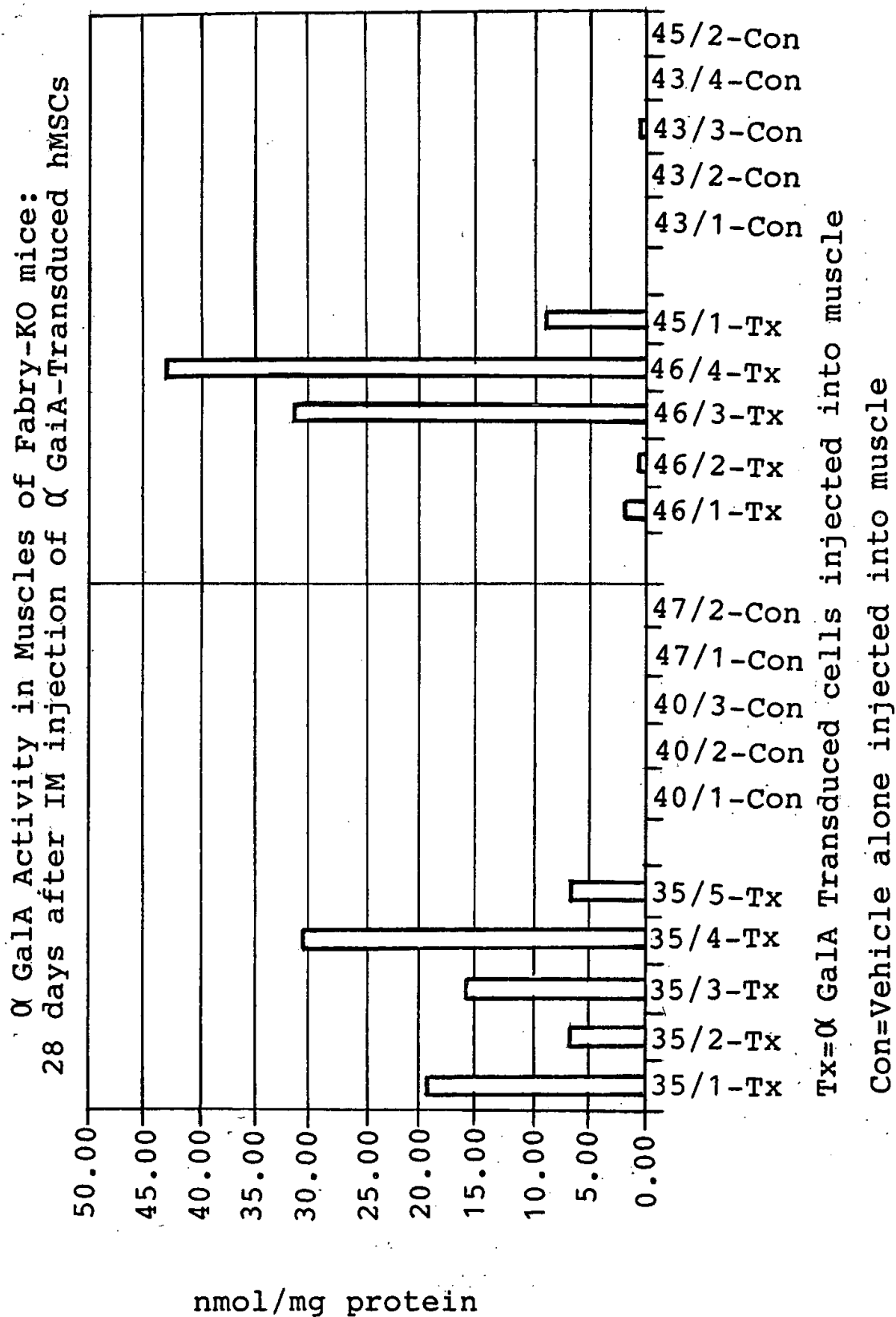


Tx = α Gala Transduced cells injected into muscle

Con = Vehicle alone injected into muscle

+

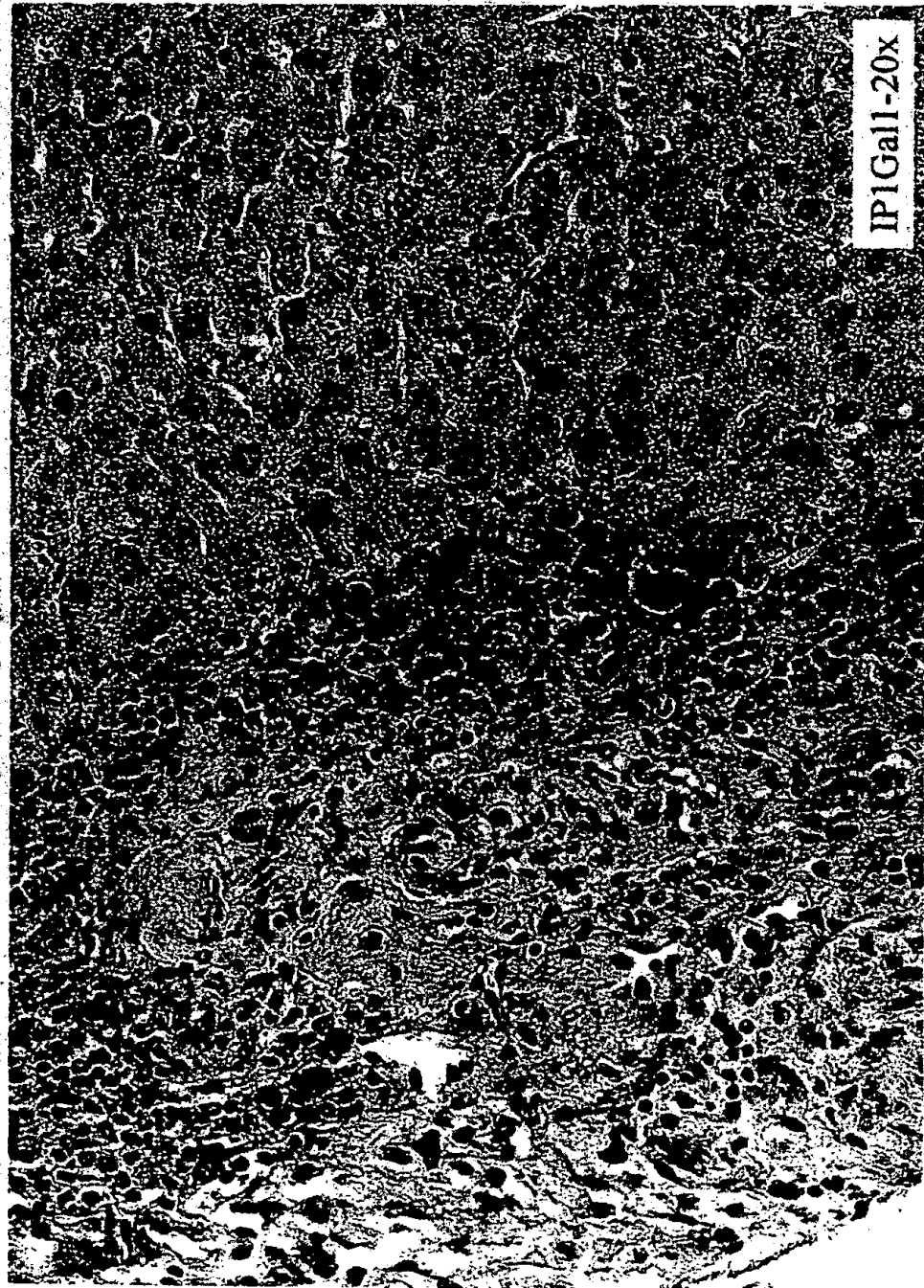
FIG. 8b



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FIG. 9a

α Gala Immunostain of Liver from KO Mouse
injected IP with α Gala-hMSCs on Cultisphers:
14 days

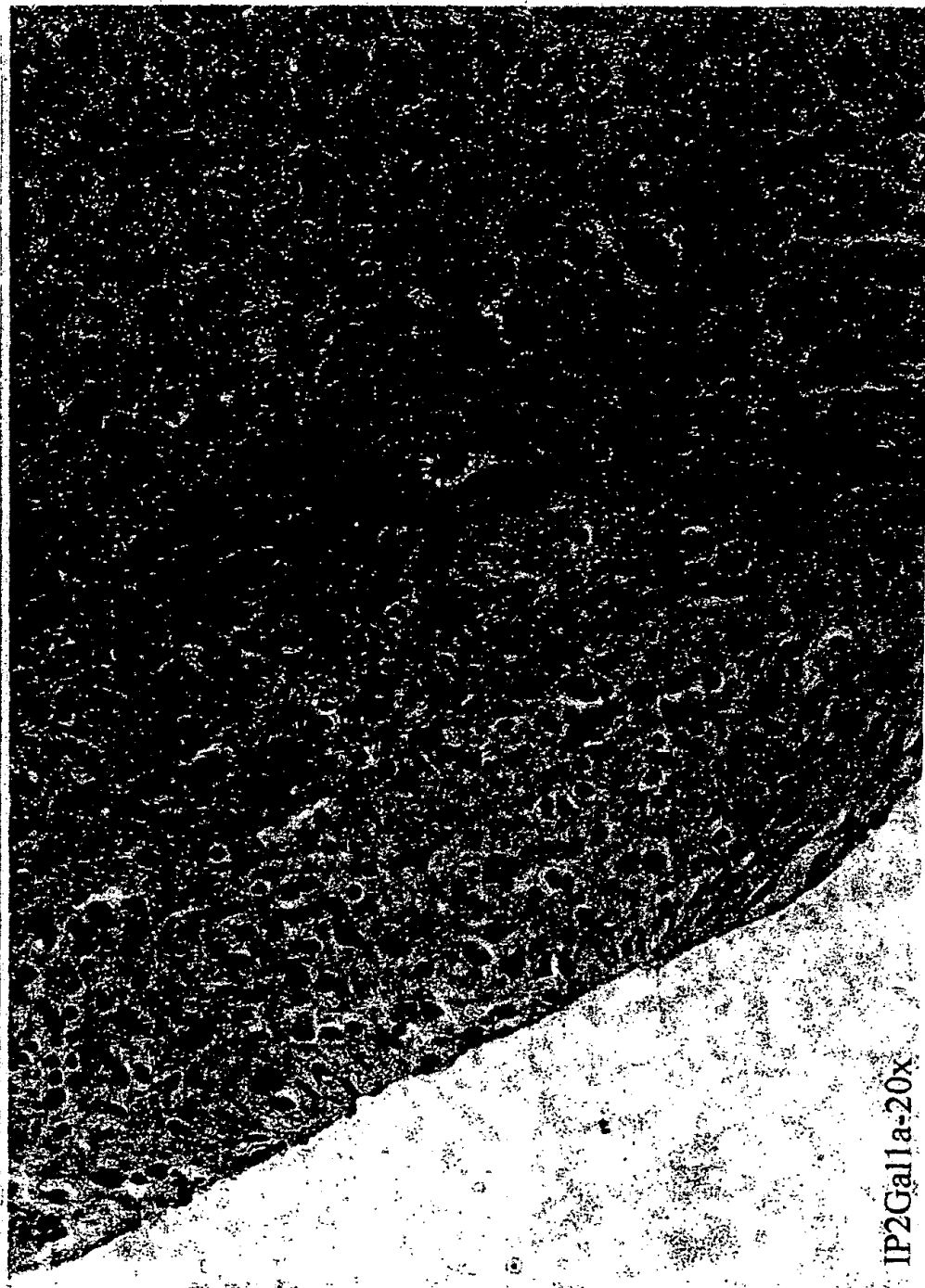


IP1Gall-20x

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FIG. 9b

α GalA Immunostain of Liver from KO Mouse
injected IP with α GalA-hMSCs on Cultispheres:
14 days



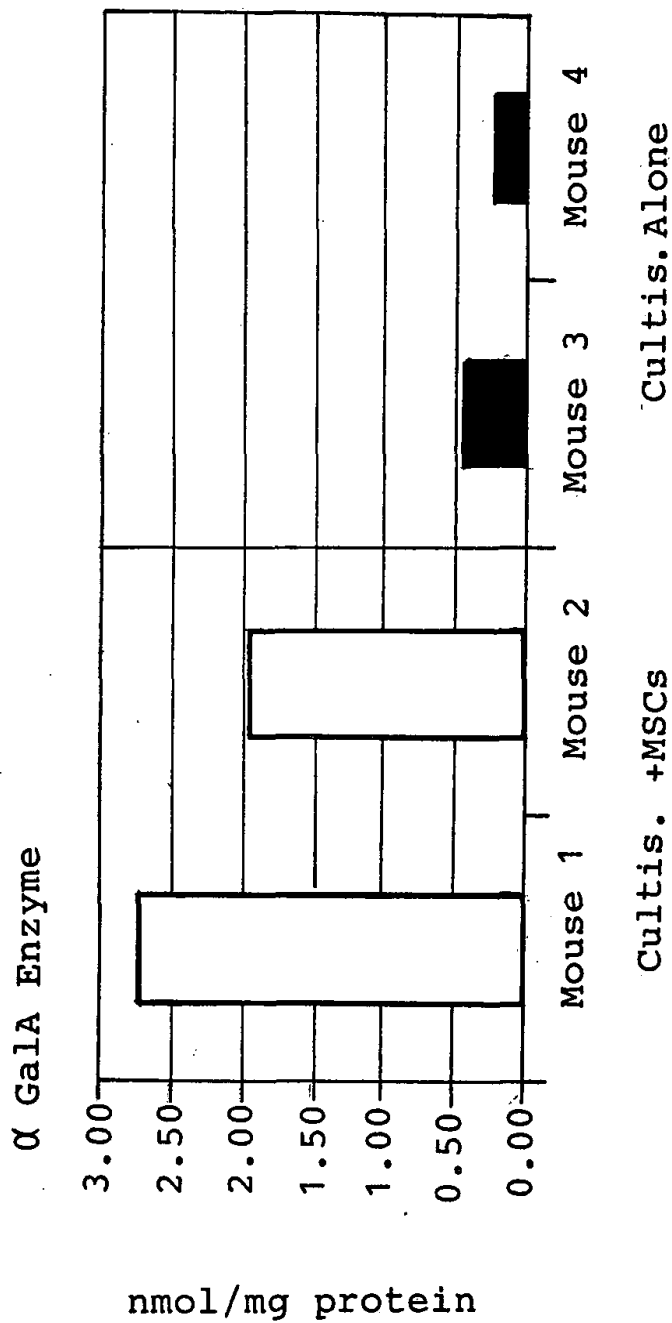
IP2Galla-20x

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FIG. 10

Intra-Peritoneal Delivery of α Gala-Transduced hMSCs
to Fabry Knock-Out Mice



Increase in α Gala is 5% of normal mouse liver activity (41 nmole/mg)

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